

STATEMENT OF HENRY A. WAXMAN

AT PRESS CONFERENCE

ON APPROVAL OF PIMOZIDE

AUGUST 7, 1984

GOOD MORNING. THIS PRESS CONFERENCE IS MORE THAN AN ANNOUNCEMENT OF THE APPROVAL OF A NEW ORPHAN DRUG. TODAY WE ARE TURNING THE PAGE IN THE STORY OF A YOUNG MAN WITH TOURETTE SYNDROME WHOSE LIFE EPITOMIZES THE PROBLEMS FACED BY AMERICAN CITIZENS WHO NEED ORPHAN DRUGS.

THE STORY IS ABOUT ADAM SELIGMAN.

IN MARCH OF 1980 ADAM'S MOTHER CALLED ME TO TELL ME THAT ADAM'S PHYSICIAN HAD JUST RETURNED FROM CANADA WITH A SIX MONTH SUPPLY OF A DRUG CALLED PIMOZIDE. WHILE THE DRUG WAS APPROVED IN CANADA, IT HAD BEEN CONFISCATED AT THE LOS ANGELES AIRPORT BECAUSE IT WASN'T APPROVED IN THE UNITED STATES. HIS MOTHER SAID TO ME, "MR. WAXMAN, I HAVE ONLY EIGHT DAYS OF PIMOZIDE LEFT FOR ADAM. WHAT ARE YOU GOING TO DO ABOUT IT?"

UNFORTUNATELY, THERE WAS LITTLE I COULD DO FOR HER THAT DAY. BUT

I SOON BEGAN A SERIES OF CONGRESSIONAL HEARINGS AT WHICH ADAM AND OTHER COURAGEOUS INDIVIDUALS WITH RARE DISEASES OPENED THEIR LIVES TO THE AMERICAN PEOPLE IN HOPES THAT THEY, AND THE CONGRESS, WOULD UNDERSTAND AND HELP.

DURING THOSE HEARINGS, IT BECAME APPARENT THAT THE PROBLEMS OF ORPHAN DRUG DEVELOPMENT ARE NOT EASILY OVERCOME. OUR SCIENTISTS HAVE NOT UNLOCKED THE SECRETS WHICH WILL SOMEDAY EXPLAIN THESE DEVASTATING RARE DISORDERS, LIKE TOURETTE SYNDROME. THE FOOD AND DRUG ADMINISTRATION AND THE NATIONAL INSTITUTES OF HEALTH ARE NOT IN THE BUSINESS OF ORPHAN DRUG DEVELOPMENT. THE COST OF ORPHAN DRUG DEVELOPMENT CAN BE SUBSTANTIAL. OUR PRIVATE, FOR PROFIT PHARMACEUTICAL COMPANIES CANNOT MAKE ANY MONEY, AND OFTEN CANNOT COVER THEIR COSTS, FOR ORPHAN DRUGS BECAUSE SO FEW PEOPLE WILL USE THEM.

THE LEGISLATIVE PATH TO ENACTMENT OF THE ORPHAN DRUG ACT WAS LONG AND, OFTEN TIMES, QUITE DISCOURAGING FOR ITS ADVOCATES. BUT ADAM AND SO MANY OTHERS, LIKE ABBEY MEYERS AND MARJORIE GUTHRIE, KEPT COMING BACK WITH SUGGESTIONS FOR LEGISLATION. FINALLY IN DECEMBER 1982, A CONSENSUS EMERGED THAT WAS SUPPORTED BY BOTH THE VOLUNTARY DISEASE ASSOCIATIONS LIKE THE TOURETTE SYNDROME ASSOCIATION, AND PHARMACEUTICAL COMPANIES.

NOW NINETEEN MONTHS LATER, WE HAVE EIGHT ORPHAN DRUGS APPROVED, 38 UNDER DEVELOPMENT, AND ANOTHER 30 BEING CONSIDERED FOR TESTING.

TODAY REPRESENTS A TRIUMPH OF PERSEVERENCE AND GREAT PERSONAL

STRENGTH FOR ADAM SELIGMAN AND THE MILLIONS OF OTHER AMERICANS WHO SUFFER FROM RARE DISEASES.

TODAY IS ALSO A TRIBUTE TO ABBEY MEYERS AND THE TOURETTE SYNDROME ASSOCIATION, MR. JACK O'BRIEN AND MCNEIL PHARMACEUTICALS, AND SECRETARY HECKLER, THE DEPARTMENT OF HEALTH AND HUMAN SERVICES AND THE FOOD AND DRUG ADMINISTRATION.

MR. O'BRIEN, I WANT TO CONGRATULATE YOU AND ALL THE EMPLOYEES AT MCNEIL FOR YOUR COMMITMENT TO PEOPLE WITH TOURETTE SYNDROME.

SECRETARY HECKLER, I ALSO WANT TO CONGRATULATE YOU, DR. BRANDT, DR. FINKEL AND OTHERS IN YOUR DEPARTMENT FOR YOUR OUTSTANDING EFFORTS IN IMPLEMENTING THE ORPHAN DRUG ACT AND SECURING COMMITMENTS FOR THE DEVELOPMENT OF SO MANY ORPHAN DRUGS.

AND, MOST IMPORTANTLY, I WANT TO RECOGNIZE ABBEY MEYERS. THOSE OF US WHO WORK WITH YOU ADMIRE AND RESPECT YOU FOR YOUR TIRELESS EFFORTS ON BEHALF OF ALL PEOPLE WITH RARE DISEASES. IT ONLY TAKES A MOMENT WITH YOU, ABBEY TO KNOW THAT YOUR DEDICATION COMES FROM YOUR HEART.

WE HAVE DONE SO MUCH WITH THE ORPHAN DRUG ACT; BUT WE MUST DO MORE. WE NEED GREATER APPROPRIATIONS FOR ORPHAN DRUG RESEARCH. WE NEED THE INTERNAL REVENUE SERVICE TO PROMULGATE REGULATIONS SO THE TAX CREDIT IN THE ORPHAN DRUG ACT CAN BEGIN. WE NEED THE NATIONAL COMMISSION ON RARE DISORDERS, WHICH I PROPOSED IN MY BILL TO REAUTHORIZE THE NATIONAL INSTITUTES OF HEALTH. THE COMMISSION WOULD

STUDY THE WAY WE ARE FUNDING RARE DISEASE RESEARCH.

WE HAVE MADE AN EXCELLENT BEGINNING WHICH HAS CREATED HIGH EXPECTATIONS. OUR GOAL IS TO SUSTAIN THIS EARLY SUCCESS, BECAUSE THE PRICE OF FAILURE IS TOO GREAT. THE IMMEASURABLE SUFFERING ENDURED BY THE MILLIONS OF AMERICANS WITH RARE DISEASES CAN BE AVERTED; BUT ONLY IF WE CONTINUE TO COMMIT OUR RESOURCES, OUR IMAGINATION AND OUR DETERMINATION.